



## ANNUAL REPORT 2011

## PRESIDENT'S REPORT

MPN Research Foundation's primary mission is to stimulate original research in pursuit of new treatments and eventually a cure for polycythemia vera, essential thrombocythemia and myelofibrosis. In addition MPN Research Foundation promotes collaboration in the scientific community to accelerate MPN research, and serves as a powerful advocacy group for patients and their families.

In 2011 the MPN Research Foundation continued direct support to critical research in the MPNs while building infrastructure to allow the Foundation to expand its impact on behalf of MPN patients. Some of our notable achievements this year include:

- Began funding 6 major new research grants (3 Established and 3 New Investigators) as identified in a grant review process at the end of 2010.
- Officially changed the name of the foundation to the MPN Research Foundation, to correspond with changes made by the World Health Organization in categorizing blood diseases.
- Designed and implemented a new website, to serve as a gateway to information about MPNs, the Foundation, and other organizations serving the MPN community which we recognize as important partners.
- Co-Hosted with CR&T the bi-annual Patient Symposium in New York City, attended by over 250 patients and other constituents.
- Developed and implemented a new physician education event in partnership with the Aplastic Anemia MDS International Foundation. This program, named the Chicago Rounds, will continue to educate clinicians in the Chicago area and will serve as a model for similar programs in other cities in upcoming years.
- Maintained the Better Business Bureau's Wise Giving seal of approval for charities.

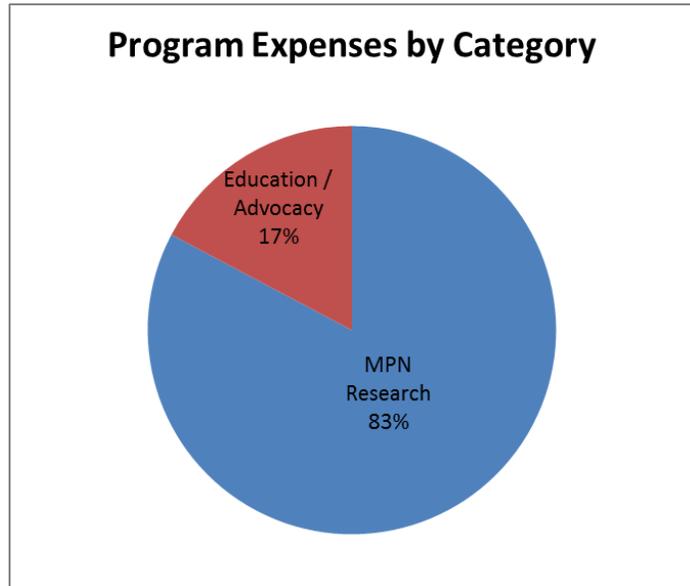
While our priority remains focused on funding innovative and accountable research we also continue to expand our efforts to educate and empower the MPN community of patients, family members, doctors and researchers. Funded by patients, for patients, we will continue to work with all members of the MPN patient community to empower patients and improve their prognoses and quality of life.

Sincerely

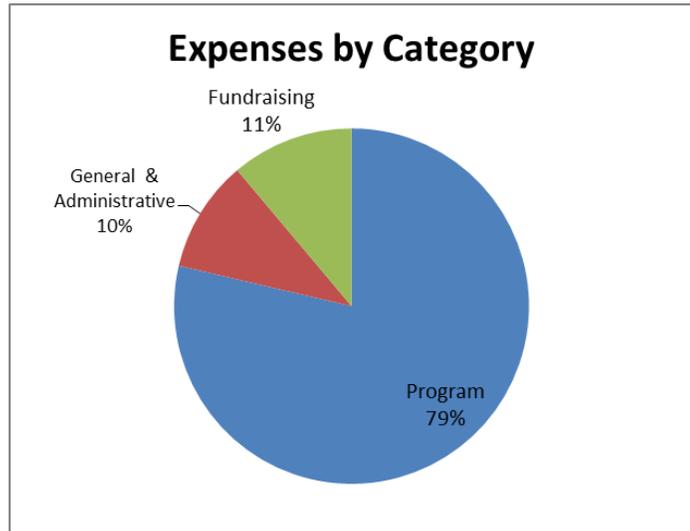
Barbara Van Husen  
President

## REVENUE AND EXPENSE FOR 2011

In 2011, while our priority remained on funding innovative MPN research, we also continued to fund education and advocacy. Patient symposia, the Rounds program for doctors, our newsletter, website and cancer center visits are vital components of our programs.



The MPN Research Foundation programs are overseen by three paid employees and two volunteer board members. As such, we strive to deliver the maximum impact for minimum cost. In 2011 we increased our efficiency, providing grant funding and educational services while holding overhead down compared to 2010.



## STATEMENT OF ACTIVITIES

THE MPN RESEARCH FOUNDATION						
STATEMENT OF ACTIVITIES						
YEARS ENDED NOVEMBER 30, 2011 AND 2010						
	2011			2010		
	Unrestricted	Temporarily Restricted	Totals	Unrestricted	Temporarily Restricted	Totals
<b>REVENUES AND OTHER SUPPORT</b>						
Contributions	1,024,773	283,550	1,308,323	1,055,623	177,900	1,233,523
Investment income	7,452		7,452	14,065		14,065
Net assets released from restrictions	314,421	(314,421)		94,281	(94,281)	
Total Revenues and Other Support	1,346,646	(30,871)	1,315,775	1,163,969	83,619	1,247,588
<b>EXPENSES</b>						
Program	1,081,636	-	1,081,636	1,124,538	-	1,124,538
General and administrative	140,309	-	140,307	181,730	-	181,730
Fundraising	153,269	-	153,269	146,692	-	146,692
Total Expenses	1,375,214	-	1,375,212	1,452,960	-	1,452,960
<b>CHANGE IN NET ASSETS</b>	(28,568)	(30,871)	(59,437)	(288,991)	83,619	(205,372)
<b>NET ASSETS -</b>						
Beginning of year	418,863	450,919	869,782	707,854	367,300	1,075,154
End of Year	390,295	420,048	810,345	418,863	450,919	869,782

## PROGRAM SERVICE ACCOMPLISHMENTS IN 2011

2011 was a year of major breakthroughs in MPN science, and saw the advancement of clinical trials that resulted, by year end, in the first commercially-available, FDA-approved treatment for myelofibrosis. Additional drugs are close behind in the drug development pipeline. The MPN Research Foundation is proud of its contribution to this progress, and we continue to look for ways to accelerate the time between discovery and treatment. This report summarizes the achievements made and the new opportunities identified in 2011 by the MPN Research Foundation on behalf of all MPN patients.

### Research

In 2011 the Foundation began funding for 8 new **academic research grants**. Each research project was selected for the direct impact it could have on patients, either in the short term or by opening new avenues for productive research in the future, and for its potential to leverage the Foundation's limited funds by producing, over time, benefits far exceeding the initial investment. Highlights of these projects include:

#### \$150,000 Established Investigator Awards

- Dr. Shaoguang Li, at the University of Massachusetts Medical School, is trying to confirm the identification of the gene Alox5 as a target for the treatment of polycythemia vera, one of the three classic MPNs. Alox5 is a promising target, because its presence is essential for the development of a related blood disease, chronic myelogenous leukemia; its loss prevents the disease from developing.
- Dr. Robert Kralovics, at the Center for Molecular Medicine, Austrian Academy of Science, is attempting to decipher the genetic complexity of myeloproliferative neoplasms through genome sequencing.
- Dr. Benjamin Ebert and Dr. Ross Levine, at Harvard Medical School and Memorial Sloan Kettering Cancer Center, will use whole genome sequencing to identify variant forms of genes that contribute to MPD pathogenesis.

#### \$75,000 New Investigator Awards

- Dr. Toshiaki Kawakami, at the La Jolla Institute for Allergy and Immunology, is studying a series of genes whose absence in mice is known to cause tumors and myeloproliferative neoplasms. He hypothesizes that the same thing happens in humans, and if it does, the discovery could lead directly to new therapeutic targets for MPN drug development.
- Dr. Wei Tong, at the University of Pennsylvania School of Medicine, is trying to determine the ways in which a protein called LNK downregulates JAK signaling. JAK2 is basically an on-off switch whose malfunction is present in many MPNs. LNK normally regulates the JAK2 switch to prevent myeloproliferation; mutated versions fail to turn off the signaling.

- Dr. Saghi Ghaffari, at the Mount Sinai School of Medicine, is investigating a different signaling mechanism whose failure may be responsible for myeloproliferation. This is important because the JAK2 mutation is not present in all MPN patients; there must be at least one other mutation to account for those cases.

Additional Grants of \$50,000 each

- Dr. Robert I. Handin, at the Brigham and Women's Hospital within the Harvard School of Medicine, is testing HDAC Inhibitors and Red Cell Proliferation in Zebrafish Embryos Expressing Human JAK2V617F.
- Dr. Ruben Mesa, at the Mayo Clinic Scottsdale, has put together a survey which seeks to validate the use of the Myeloproliferative Neoplasm Symptom Assessment Form Diary to Assess Symptomatic Pains in Patients with Polycythemia Vera and Post Polycythemia Vera.

In addition to the items above, the Foundation began to design major new research initiative which will, we believe, open new doors to MPN research and can have a major impact on the lives of MPN patients. Based on patient input, the Foundation began in 2011 to study the effect of bone marrow fibrosis on MPN patient prognoses, and with the input of our scientific advisors developed a new grant program called The MF Challenge, to be initiated in early 2012. The goal of the MF Challenge is to discover the cause(s) and potential reversal of bone marrow fibrosis, using emerging developments in other types of fibrosis as a base and a model. To fund this new initiative, the Foundation secured first-year funding from individual donors and established a partnership with the Leukemia & Lymphoma Society (LLS) which will match that funding. A Request for Proposal for innovative concept grants to be awarded in mid-2012 has been developed jointly by the Foundation and LLS and will be issued in January, 2012.

### **Education and Advocacy**

The Foundation co-hosted a patient symposium in New York City with New York-based Cancer Research & Treatment (CR&T). This bi-annual symposium, which brings top researchers to discuss the state of the art of MPN science was attended by over 250 patients, family and supporters.

As part of our effort to educate increasing numbers of doctors on the most current MPN research and treatment options, The Foundation developed and implemented a new physician-education program, in partnership with the Aplastic Anemia MDS International Foundation (AAMDSI). The program's first event, titled the Chicago Rounds, brought university and community physicians together to hear a keynote speaker as well as specific cases presented by participation research organizations in Chicago. Based on the success of the first event, several more are being planned for Chicago in 2012, and will serve as a model for similar programs in other cities in upcoming years.

The Foundation remains committed to educating patients through its free semi-annual newsletter, as well as assisting with coordination of patient support groups in the U.S. and abroad. In addition, the Foundation actively seeks to connect with physicians

worldwide to provide the information and support they need for both clinical practice and patient support.

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